Rettsyndrome.org
ACCELERATING RESEARCH. EMPOWERING FAMILIES.

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Our Research Strategy Delivers

Having recently joined Rettsyndrome.org, I am humbled and grateful for the opportunity to serve this very special community.

My 30 years as a health care administrator has taught me that medical solutions to even the simplest condition can be complicated and challenging. For example, there are about 75 drugs to treat something as "simple" as high blood pressure. This large number of drugs is necessary because *physicians need to be able to work with each patient and identify a drug that will work for that individual at that specific point in time.* So imagine how many different therapies, drugs, and interventions will be needed to effectively treat the very complex, genetic, neurodevelopmental disorder that is Rett syndrome! While we would all love the idea of a single dose cure, this disorder is far too obstinate for that.

It is going to take a village of people, armed with a "cabinet" full of biologics and therapies, to eradicate Rett syndrome. The research needed requires an attentive and directed approach. Rettsyndrome.org clearly understands that. We remain resolute in funding research to advance the field of pharmaceutical intervention (drug therapies, gene therapy, and protein replacement therapies) coupled with work re-establishing neural networks. Both are needed to undo the effects of and deliver a cure for Rett syndrome.

So how do we make sure that the research we fund is most likely to be effective?

Each year, Rettsyndrome.org assembles with a group of trusted scientific experts and advisors to identify the gaps in research so that we can understand what research we need to support. We ask experts in the genetics of Rett syndrome to identify which areas need focused attention. We ask translational scientists to identify what is needed to move our basic science discoveries into the clinics. We ask clinical researchers to identify how we can improve clinical trial environments. We ask experts in neural networks to help us design research programs focused on establishing vital neural networks that were never fully developed. We take all of this information and issue our Request for Applications (RFAs).

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These RFAs are released worldwide and allow all scientists to submit their most promising ideas. These ideas are evaluated by peers and the best are funded. Our approach is unique because we look at all applications. We do this because we believe that the best ideas can come from anywhere and anyone, even scientists that are new to us. Many of the top researchers in Rett syndrome got one of their first grants from Rettsyndrome.org. We recently held our meeting for the 2018-2019 research cycle and are very excited about our future direction.

We deliver research with results.

Our process works and our success is unmatched in Rett syndrome research. Our 44 million dollar investment in research is the *only one that has delivered* a drug that is entering the final stages of human clinical trials, with three more close behind. Our research strategy is supported by the experts and it is proven. We have led the way in supporting research from basic science to neuro-habilitation, and in bringing compounds through drug discovery and into clinical trials. We have partnered with clinics to develop the largest and most comprehensive Natural History Study.

There is much left to do, but we are encouraged by how far we have come and are grateful to the families, clinics, researchers and donors who support us.

With hope and gratitude,

Melissa Kennedy
Executive Director